Oxbryta® (voxelotor)
(Oral)

Document Number: IC-0516

Last Review Date: 01/05/2023
Date of Origin: 01/03/2020
Dates Reviewed: 01/2020, 01/2021, 01/2022, 01/2023

I. Length of Authorization

Coverage will be provided for 6 months initially and may be renewed annually thereafter.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Oxbryta 300 mg tablets: 5 tablets daily
- Oxbryta 500 mg tablets: 3 tablets daily
- Oxbryta 300 mg tablets for oral suspension: 5 tablets daily

B. Max Units (per dose and over time) [HCPCS Unit]:

- 1500 mg daily

III. Initial Approval Criteria [1]

Coverage is provided in the following conditions:

- Patient is at least 4 years of age; **AND**

Universal Criteria [1]

- Hemoglobin (Hb) lab values are obtained within 30 days of the date of administration (unless otherwise indicated); **AND**
- Will not be used in combination with crizanlizumab (Adakveo) or L-glutamine (Endari); **AND**
- Patient will avoid concomitant therapy with strong or moderate CYP3A4 inducers (e.g., rifampin, carbamazepine, St. John's Wort, phenobarbital, etc.), or if therapy is unavoidable, the patient will be monitored closely for adverse reaction and/or dose modifications will be implemented; **AND**

Sickle Cell Disease † Φ [1-3,5]

- Patient has a confirmed diagnosis of sickle-cell disease, of any genotype (e.g., HbSS, HbSC, HbS/beta^0-thalassemia, HbS/beta$^+$-thalassemia, and others) as determined by one of the following:
  - Identification of significant quantities of HbS with or without an additional abnormal ß-globin chain variant by hemoglobin assay; **OR**
  - Identification of biallelic *HBB* pathogenic variants where at least one allele is the p.Glu6Val pathogenic variant on molecular genetic testing; **AND**
- Patient had an insufficient response to a minimum 3-month trial of hydroxyurea (unless contraindicated or intolerant); **AND**
- Patient experienced one or more vaso-occlusive crises (VOC)***** in the previous year despite adherence to hydroxyurea therapy; **AND**
- Patient has symptomatic anemia with a baseline hemoglobin (Hb) between ≥ 5.5 g/dL to ≤ 10.5 g/dL prior to start of therapy; **AND**
- Other causes of anemia (e.g., hemolysis not attributed to SCD, bleeding, vitamin deficiency, etc.) have been ruled out

*****VOC is defined as an event prompting either a visit or outreach to the provider which results in a diagnosis of VOC being made necessitating subsequent interventions such as narcotic pain management, non-steroidal anti-inflammatory therapy, hydration, etc.

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria [1,3]

Coverage can be renewed based upon the following criteria:

- Patient continues to meet the universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: severe hypersensitivity reactions, etc.; **AND**
- Disease response as evidenced by an increase in hemoglobin of >1 g/dL from baseline

V. Dosage/Administration [1]

Indication	Dose
Sickle Cell Disease	<u>Pediatric Patients 4 years to <12 years of age</u> • ≥40 kg: 1,500 mg orally once daily • 20 kg to <40 kg: 900 mg orally once daily • 10 kg to <20 kg: 600 mg orally once daily *****NOTE:** Select the appropriate product (Oxbryta tablets or Oxbryta tablets for oral suspension) based on patient's ability to swallow tablets and patient*

	weight. Oxbryta oral tablets are available as 300 mg or 500 mg and Oxbryta tablets for oral suspension are available as 300mg. **Adults and Pediatric Patients ≥12 years of age** • 1,500 mg orally once daily

VI. Billing Code/Availability Information

HCPCS:

- J8499 – Prescription drug, oral, non chemotherapeutic, nos

NDC:

- Oxbryta 300 mg tablets: 72786-0102-xx
- Oxbryta 500 mg tablets: 72786-0101-xx
- Oxbryta 300 mg tablets for oral suspension: 72786-0111-xx

VII. References

1. Oxbryta [package insert]. San Francisco, CA; Global Blood Therapeutics, Inc., October 2022. Accessed November 2022.
2. Bender MA, Carlberg K. Sickle Cell Disease. 2003 Sep 15 [Updated 2022 Nov 17]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2022. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1377/.
3. Vichinsky E, Hoppe CC, Ataga KI, et al; HOPE Trial Investigators. A Phase 3 Randomized Trial of Voxelotor in Sickle Cell Disease. N Engl J Med. 2019 Aug 8;381(6):509-519. Doi: 10.1056/NEJMoa1903212. Epub 2019 Jun 14.
4. Yawn BP, Buchanan GR, Afenyi-Annan AN, et al. Management of sickle cell disease: summary of the 2014 evidence-based report by expert panel members. JAMA. 2014 Sep 10;312(10):1033-48.
5. Estepp JH, Kalpatthi R, Woods G, et al. Safety and Efficacy of Voxelotor in Pediatric Patients With Sickle Cell Disease Aged 4-11 Years: Results From the Phase 2a HOPE-KIDS 1 Study. Presentation given during European Hematology Association Congress 2021.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
D57.01	Hb-SS disease with acute chest syndrome
D57.02	Hb-SS disease with splenic sequestration
D57.03	Hb-SS disease with cerebral vascular involvement
D57.09	Hb-SS disease with crisis with other specified complication
D57.1	Sickle-cell disease without crisis
D57.20	Sickle-cell/Hb-C disease without crisis

D57.211	Sickle-cell/Hb-C disease with acute chest syndrome
D57.212	Sickle-cell/Hb-C disease with splenic sequestration
D57.213	Sickle-cell/Hb-C disease with cerebral vascular involvement
D57.218	Sickle-cell/Hb-C disease with crisis with other specified complication
D57.3	Sickle-cell trait
D57.411	Sickle-cell thalassemia with acute chest syndrome
D57.412	Sickle-cell thalassemia with splenic sequestration
D57.418	Sickle-cell thalassemia, unspecified, with crisis with other specified complication
D57.42	Sickle-cell thalassemia beta zero without crisis
D57.431	Sickle-cell thalassemia beta zero with acute chest syndrome
D57.432	Sickle-cell thalassemia beta zero with splenic sequestration
D57.433	Sickle-cell thalassemia beta zero with cerebral vascular involvement
D57.438	Sickle-cell thalassemia beta zero with crisis with other specified complication
D57.439	Sickle-cell thalassemia beta zero with crisis, unspecified
D57.44	Sickle-cell thalassemia beta plus without crisis
D57.451	Sickle-cell thalassemia beta plus with acute chest syndrome
D57.452	Sickle-cell thalassemia beta plus with splenic sequestration
D57.453	Sickle-cell thalassemia beta plus with cerebral vascular involvement
D57.458	Sickle-cell thalassemia beta plus with crisis with other specified complication
D57.459	Sickle-cell thalassemia beta plus with crisis, unspecified
D57.80	Other sickle-cell disorders without crisis
D57.811	Other sickle-cell disorders with acute chest syndrome
D57.812	Other sickle-cell disorders with splenic sequestration
D57.813	Other sickle-cell disorders with cerebral vascular involvement
D57.818	Other sickle-cell disorders with crisis with other specified complication

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Determinations (LCDs), and Local Coverage Articles may exist and compliance with these policies is required where applicable. They can be found at:

https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC